CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

California Institute for Regenerative Medicine
Patient Advocate Focus Group Meeting
July 17, 2006

On July 17, 2006, the California Institute for Regenerative Medicine (CIRM) held a focus group meeting for a number of patient advocates (representing a broad range of diseases and conditions) from throughout the state of California. This meeting was structured around a series of questions regarding the focus, goals, and values of CIRM, among other issues; the answers to those questions are summarized below. This summary is not intended to be comprehensive with respect to reporting these answers, nor does inclusion in this summary imply any commitment or endorsement by the CIRM.

INTRODUCTIONS

- Zach Hall welcomed the attendees and thanked them all for joining the discussion. He stressed that the support of the patient advocates was critical to the success of Proposition 71. He mentioned that there are patient advocates on the ICOC. He also gave an overview of the scientific strategic planning process.
  - Last October, CIRM invited scientists from around the world to participate in a forum to define the state of stem cell research and address what CIRM could do to advance stem cell research. At this conference, we discussed some of the challenges and opportunities they were facing.
  - CIRM is interviewing people from all over the world including stem cell scientists, scientific leaders, patient advocates, public policy experts, ethicists, and scientists and leaders from the private sector. CIRM plans to interview approximately 70 people.
  - CIRM has held public, scientific strategy meetings, which included speakers and discussions.
  - CIRM is having parts of two meetings of the ICOC devoted to a discussion of the mission, objectives and values for the plan.
  - CIRM is also having focus groups, of which this is the first.

- Bob Klein acknowledged the efforts of David Serrano Sewell and Susan DeLaurentis in organizing this meeting.

- Patricia Olson discussed the format for the meeting.

- A set of questions on a variety of topics has been developed and will be used as guidelines for this discussion. The discussion will be an informal and with about 15 minutes spent on each question.
- A link on the CIRM website is a direct way to contact the strategic planning team.

- David Serrano Sewell stressed that the strategic plan is very important and will guide the CIRM for many years to come. He added that this focus group is part of this ongoing dialogue.

DISCUSSION QUESTIONS

Overall Goals

1. **How would you define success for the Institute over the next ten years? What are your expectations?**

- **Participant #5:** We can gauge our success over the next 10 years if the scientific goals that have been laid out have been reached or are in the process of being attained. If scientific goals are being attained, we have something that we can measure. We can look at our goals in terms of supporting and giving a more conducive climate for cooperation with the scientific strategic plan. That will be one of our major goals.

- **Participant #3:** Success would be that stem cells or their derivatives would have cured many of the diseases that plague society.

- **Participant #12:** I would not expect a cure in ten years and am not sure if damaged cells can be repaired or not. In five years, if we inject stem cells, what would happen? Right now, anything can happen. In five years, we should know in what direction that can be controlled. My response for success is if we are getting closer to a cure in ten years.

- **Participant #4:** In ten years, I would want to see us move past basic research. We expect to see something, whether in Parkinson's or another disease. Something should happen which will in turn wipe out all the controversy we are seeing. I expect to see something real happen in ten years. I expect to see clinical trials that are ongoing or that are about to happen in ten years. I expect CIRM will be the center for stem cell research in the world. I expect that there will be collaboration between scientists that will be unheard of, which is part of what will make it work. Collaboration is extremely important, especially in obtaining stem cells. Perhaps through the Institute, scientists from all over the world would be able to use these cells and clinicians would have access to centralized research data [on these cells].

- **Participant #7:** The expectation is that there will be major breakthroughs in lots of different illnesses. We are in California and at the cutting edge, so one way to define success is through the approach we take - that isn't business as usual. California could come up with

something that would create collaboration and new models of research. We could do things differently so that we can reach our goals and cure people. We could lead the way to a research revolution, not only in stem cell research but in science.

- **Participant #8:** It's all about the concept of scientific readiness. We are not starting from a dead stop. There are things that are happening that will feed in to what we are doing. We can try to identify scientific readiness, whether it is one of the diseases or critical technology. This should happen as one of our first priorities and could affect what can happen in the first ten years. Some of the scientists working on ALS do not feel it is a false expectation to have an ALS therapy that is close to the clinic in ten years, that is, through clinical trials and close to routine use.

- **Participant #17:** In ten years, there should be at least one therapeutic intervention that has been approved, and perhaps three clinical trials in stage III. I hope that is not too optimistic, but that's what we should be able to achieve.

- **Participant #13:** Five years was the timeframe for getting genetic therapy to market - it is eleven years later, and we're still waiting. There should be some concrete markers [of progress] in a disease if we are to have optimal cures. I don't think we'll have a cure for cystic fibrosis in ten years but I am optimistic that we will have developed ways to regenerate lung tissues. Stem cell lines may serve as efficient ways to test medications.

- **Participant #13:** Another marker [for success] is how many people are coming into the field. My fear is that people would not pursue this because of a lack of funding, so we should have a marker of how many new minds are coming in. That would help everyone because researchers have different disease emphases.

- **Participant #10:** There are different aspects of stem cells when trying to understand a disease. There is so much going on. CIRM has a chance to take a leadership position to set standards within the field. We should look at the biology in more of a systems biology way to understand how a specific disease affects the whole body. We need to look at the whole system. This is an opportunity for coordinating scientists and the different ways they are approaching this. Some scientists approach the biology and some drill down to diseases.

- **Participant #2:** When you look at Alzheimer's, we've made strides in the last ten years. I see the Institute as a leader in sharing information and in collaborative efforts to allow people to make leaps in different diseases. In addition to understanding more about disease, researchers, in my experience, work in silos because they protect information so that they can publish. If they were able to share ideas and new information, we would make great leaps in the research arena. I see the CIRM as a leader in ten years in putting people in touch with each other. We can't make cures in ten years.

- **Participant #3:** I would like to see the politics removed. All this time and effort is used in the political debate. Let the scientists do the science.

- **Participant #9:** For the first 30 years of the Juvenile Diabetes Research Foundation, the focus was on basic research. More recently, they cut down the emphasis on basic research and started doing clinical research. Streamlining was important. I live in San Diego and am amazed that there is no communication from one side of the state to the other. If we could have some synergies and collaborations, that would be great. It seems to me that we should be cutting down the time for basic research so that we can have clinical research and promote synergies.

- **Participant #18:** We need to be sharing this knowledge. Cell therapies and stem cell lines can be used as tools for toxicity testing. This is a truly valuable product and mechanism to feed to the clinical side. Knowledge will help with existing therapies to slow down disease or enhance therapeutic agents that are currently available. If we don't have a system that transfers information, we will waste time that will impact the lives of patients. There is no mechanism to immediately transfer information between researchers and clinicians. Maybe we should have some interface with the medical profession such as having a scientific medical joint conference twice a year on different diseases. How can we get impact from the knowledge we gain every year?

- **Patricia Olson:** To summarize, we have heard that we need to push towards therapies, have the tools and technology in place, be a leader for collaboration, have information sharing, and make California a leader in stem cell research.

2. What are some concrete milestones (3 year, 5 year) the Institute might use to gauge its progress?

- **Participant #5:** We need to gauge it by a certain number or quantity of clinical interventions so that we can say that the accomplishments have achieved so many interventions in different diseases. We could do this rather than quantifying goals of different diseases, and we should look at reducing the cost of healthcare. There needs to be some way to measure these milestones that create justification of a continued effort by the Institute. In terms of ten years, we should be looking at what is being accomplished by CIRM that continues to justify this progress. In ten years, we don't want this Institute to fold but to continue the process and to act in a continuum. Milestones have to be something measured in terms of a continuous process.

- **Participant #7:** Why don't we throw out the concept of milestones? Milestones represent the way we have done things in the past. We should measure how things are progressing. We want to see ways to fund innovations. Scientists should be free to follow leads and their intuition and that does not follow the milestone model. We should think about how to judge progress in a different way. I'm not sure what that model is, but perhaps we can have different types of grants or look at other ways to push research forward. I would not like to throw out milestones all together, but I think we can use both approaches.

- **Patricia Olson:** It is not so much milestones for inventions but for the CIRM to measure how we're progressing. If our goal is to have X number of things in clinical trials and be recognized as a leader in stem cell research, which implies things getting done along the way. What do you expect to hear when we communicate to you the advances we've made in three years? What do you expect to hear so that you are going to know we're making progress and that we are on the right road?

- **Participant #8:** If at some point (in three to five years, with sooner being better) we are able to master the technique of somatic cell nuclear transfer, this will lead to everything else that happens.

- **Participant #2:** It is hard to talk about milestones because you don't know how much control you have over those who make those milestones occur. We should think more of the outcomes. In terms of milestones, you have to ask, "How do you get there?" In my mind, in two years, maybe we will have provided a symposium for researchers to gain information. In four years, we will have talked about collaboration that involves stem cell centers in northern and southern California. If we talk about transparency, those are things we can say. We want milestones that compel us to be a leader. What puts us there? These will be measurable things that will be transparent.

- **Participant #17:** An information portal is something that is measurable. Within two years we could have an information portal but you have to figure out what you want on it and how to make it accessible. We should have some sort of intervention in human clinical trials in five years. Then if we want additional funding, we can say this is what we have accomplished.

- **Participant #4:** The strength of CIRM is to be the innovative body without restrictions to tie us down. It is incredibly important that we put objectives down and work towards them. That is human nature. But we don't want to limit ourselves, so we've got to allow out-of-the-box thinking and make sure terminology doesn't lock us in.

- **Participant #10:** How do we keep track of what is going on outside of California? How do we bring it in and have us take part in it?

- **Participant #15:** CIRM's leadership can be demonstrated by serving as a catalyst to mature a very early industry. CIRM can help create an environment for innovative thinking. Some of the specific things are setting up cell banks and cell databanks, facilitating access to information for researchers, and supporting training in techniques for researchers. Those kinds of things will help a broad number of researchers do their work better and more efficiently. We should lay the landscape that can allow them to bear more fruit down the road. We should be tracking specific milestones such as, "How many papers are being published?" "Who is doing stem cell research right now?" "How much money is being spent?" These should be tracked throughout the year.

- **Zach Hall:** Does everyone understand nuclear transfer and why it's important?
  - Nuclear transfer involves taking stem cells and growing them in a lab to become specialized cells, but instead of using embryos from IVF clinics, which are genetically not diverse, cell lines can be created (with broader genetic backgrounds) by taking an unfertilized egg, removing the nucleus, and replacing it with another nucleus from an adult cell. When you then make a stem cell line, it has the genes of that person from whom the nucleus is derived.
  - The power of this approach is that if someone has a hereditary form of ALS or Parkinson's or Alzheimer's, you can create a stem cell line with those genetics and study why those genes confer susceptibility to the disease.
  - If we have a human cellular model of a disease, you can compare that with normal cells and find out what was different. You could think about new therapies and even screen drugs.
  - This is not a conventional use of stem cells for therapeutics; rather it is a tool for discovery.

- **Participant #10:** Looking at Pre-implantation Genetic Diagnosis (PGD) and creating lines for those diseases, particularly pediatric ones, would be useful and potentially helpful to understand adult degenerative disorders.

- **Participant #1:** We should try to figure out an easy way to explain this to legislators. We need to talk about this in a more understandable way.

Values

3. **What do you think are the essential features and values that should be embedded in the strategic planning process, and more broadly in the scientific funding process?**

- **Patricia Olson:** I heard collaboration. Are there other values?

- **Participant #5:** We need to have some values that measure success. From a value point of view, success has to be something that shows that a true benefit has been achieved or accomplished - not just in the therapeutic context of a trial, but that an actual benefit has been accomplished or quantified. If we want to call that clinical accountability, we could use that to define a value.

- **Patricia Olson:** When you say benefit, what do you mean?

- **Participant #5:** The benefit is showing that it has been able to alleviate some symptom or condition relative to a disease.

- **Participant #14:** Everyone talks about transparency. It has to be more active. Transparency seems passive to me. There needs to be more active transparency, specifically communication between researchers and clinicians. Information must be communicated to all

interested parties including patients. There should be active communication as opposed to transparency.

- **Participant #7:** What do I get for my investment? This should be a value - communicating to all of California that this is a good investment that's paying off. As citizens of California, we want to know what are they are getting for their investment. I'm not sure how we can do that. It's about accountability and communication to the citizens.

- **Participant #12:** The scientists are also accountable to the Institute.

- **Participant #8:** We would like to see CIRM take the position that it gives priority to evaluating proposals or seeking proposals that only CIRM can do and that NIH cannot do. This includes working with human embryonic stem cells as opposed to mouse cells. That's difficult and so far no one is really doing it. People will avoid it because it is difficult. We can do this because CIRM can fund it. We can encourage it and solve the technology of it.

- **Participant #2:** This is a controversial area for some people, so the value of integrity is important. It is important that CIRM operates with integrity and be above reproach.

- **Participant #17:** If we can get agreement with the principal investigator to be open with communication in terms of what works and what fails, this would speed up progress. Right now, only what has worked has been published, not what has failed. Failures can speed up our research, too.

- **Participant #1:** In terms of opportunities to encourage collaboration, we need to focus on partnerships, not competitiveness. It is not about simply encouraging those things, but insisting on them. We should set the bar high in all of these areas. There should be a sense that sharing is important to progress and success.

- **Participant #12:** CIRM-funded scientists should be open to new findings from other scientists.

- **Participant #4:** Collaboration allows scientists from outside a given laboratory to come together. Collaboration does not need to be forced - it's an opportunity. If you offer space and equipment where scientists can come together outside their labs, this will be an opportunity.

Research

4. **What is the best way to foster stem cell research that achieves treatments and diagnostics? Should CIRM target specific diseases? If so, how would CIRM decide how and when to fund disease-targeted research? And/ or should CIRM sponsor more broadly based initiatives within which a variety of disease can be accommodated?**

- **Participant #10:** My foundation went though this. We brought together 40 stem cell scientists to find out what is happening in the field. After we got that information, we drilled down to our focus to see where we understood the mechanisms and where we could play a role. We also looked at the neurological piece. Something like cerebral palsy won't get to clinical trials but Tay-Sachs, Batten's disease, and Canavan's disease will get to clinical trials faster. Some of these pediatric disorders are easier to look at since patients don't have sixty years of environmental issues to deal with. These diseases are developmental and they are also correlated to adult conditions. Maybe we need to look more at the developmental perspective and see where we can have a success in clinical trials.

- **Participant #5:** I represent over 1 million people in California who are affected with [an organ] disease. I would be more in favor of more broadly sponsored initiatives in which more broadly based diseases should be targeted. All diseases should be judged by the degree of suffering. The amount of money should not be targeted toward diseases that have the most number of people afflicted, but have the most suffering among those who have that illness. Diseases such as Parkinson's and ALS are funded much less than diseases such as AIDS. Those that cause greater suffering, such as diabetes, should get more equitable funding. It should not be based on the number of people afflicted with the disease. The degree of suffering must be accounted for. We should do something that will provide benefits broadly.

- **Participant #3:** In terms of low hanging fruit, what is going to get some progress sooner? Whatever can get the momentum going.

- **Participant #4:** What it comes down to is the basic science. We have to go to the science.

- **Participant #12:** I think we should have a little of both, but lean towards more broadly based initiatives, recognizing that we have to have some success out of the gate. I am leaning towards understanding more about stem cells and then going forward.

- **Participant #6:** "What do you think has the most promise?" This is the hardest question to answer. As a community, we have to think if "low hanging fruit" is a concept we're OK with, and if we are, we need a better term for it. Whether it about subjective pain or watching loved ones suffer, or whatever it is we use as a measuring stick, we have to remember that ultimately, it will be 15 stem cell scientists on the Grants Working Group that will make that first recommendation and a 29 member board making the funding decisions. But the front end are these scientists.

- **Participant #10:** We should start broadly. We should start at where we know enough of the science and push it.

- **Participant #8:** I don't think it is an either / or. We know that there are a group of diseases such as one where neurons die. There are groups of diseases that have this in common. For example, we can look at neuron death to determine what the signals are and how to prevent it. This would encompass a group of diseases that fall into this category. It's not all based on the scientific committee of 15, because the call goes out to researchers based on the specifics of the call and what's in the strategic plan.

- **Participant #13:** It would be a mistake to start with an either/or approach. In the beginning, it has to be an "and" approach. There are diseases where people are focusing on clinical research but we need broad-based initiatives. We need to see that each funded project is something leading to the next step. It seems that so much funding goes to studies and then ends up sitting on a shelf. There are all building blocks for the end.

- **Participant #17:** There is a need to do the science, but not to get so much into the basic research. We should find a way to target the research to get success. We have to consider numbers. What can we do to be successful and still do the science? If we can grow those cell lines and come up with therapeutics, we can potentially get new funding down the line.

- **Participant #2:** To me, "low hanging fruit" means we look at things with an opportunistic approach. We start by looking at the themes that produce trends that have potential to rise to the top. We fund the things that have potential to be replicable and move to the next step in a way that leads to potential new development, whether or not someone else takes it and runs with it. We need to look at what has the most potential to move forward and take it to another level. We have to do both and look at it from the perspective of what has the most potential to go forward. That is going to be a subjective decision.

- **Participant #15:** Maybe part of the application process and part of the value can be where a research proposal fits in the continuum. What is the purpose? What does this lead to? It's not just about an interesting question that gets published. Maybe we should focus on tools and techniques (i.e. SCNT) and have the scientists run with it. We should equip them to allow them to be more creative and effective.

- **Participant #9:** We should attack autoimmune disease as a group versus problems that maybe come from external sources. All our diseases are inter-related. I know a diabetes researcher studying regeneration of the pancreas and he has found a breakthrough in pancreatic cancer. I do a lot of work with lymphoma and this group is using the same monoclonal antibodies as are being used in diabetes. The body is so complex and yet so interrelated with all these diseases. She would hate to see this be too disease-targeted because we don't know where the answer will come from. The communication is critical - we have to know what's going on in the state and around the world.

- **Participant #10:** This goes back to systems biology. We don't know which part of the body these answers come from.

5. Should the CIRM fund research that also could be funded by NIH?

- **Patricia Olson:** Someone mentioned that there is a mandate in Proposition 71 to fund research that is not funded by the NIH. But the proposition also provides for the funding of stem cell research that has great potential for therapies and cures that is unlikely to receive timely or sufficient federal funding. There is a lot of NIH eligible research that could perhaps be helpful. How do we fit that in?

- **Zach Hall:** We're talking about things that may not get enough funding.

- **Patricia Olson:** I also want to remind the group that the NIH is not giving much money to this area [stem cell research]. Also, the NIH budget is not growing - it is flat or decreasing.

- **Participant #8:** Once the strategic plan is set and if there is agreement of the priorities, it almost does not matter where the funding is coming from. We need to make sure that the pieces that fit in the strategic plan are being funded. If it can be funded by NIH, that is fine, but given the status of funding that NIH is giving, we know this is going to be a problem.

- **Participant #7:** My first reaction was no, but that's not realistic. The strategic plan makes sure that what we are doing falls into our values and that we are not duplicating what's been done before.

- **Patricia Olson:** I want to remind everyone that reproducibility is an important component of scientific research. We are always building upon and assuring ourselves that the data is real and valid. That is an important component of research.

- **Participant #8:** There are other funding sources for many of these diseases and techniques. All these have to be taken into account when decisions like this are made.

- **Participant #4:** We have to be careful not to make it so structured and so rigid. There has to be flexibility.

- **Participant #3:** We have to remember that NIH funding represents a particular stage in research funding. It's a stage and we have to look at a different way to cut it.

- **Participant #5:** I agree that if there is something constructive that is NIH funded that may be useful to us, we should definitely have the possibility to use that technology because knowledge should not be exclusive. I feel that there should be a way we can use NIH funded project outcomes if it is useful to us, but there is no point in us duplicating it.

- **Patricia Olson:** Could CIRM also fund research that could theoretically be funded by NIH?

- **Participant #5:** If that information was useful to us, there should be a point that we include that process in our research. We should be able to do that research because it is constructive to our goals.

- **Participant #13:** I agree that there should be funding for NIH-funded research, but the priority should be for those not getting NIH funding. There is a mandate from the people that we want to do something different and I would hate to see that getting lost. If it's a great project the project shouldn't be lost either, but we should remember our priorities.

- **Participant #12:** That's CIRM's niche - the risky innovative research (versus minimal risk research). We do need results out of the gate, but we also need to remember this is our niche.

6. **What about research that is risky and innovative vs. research that has minimum risk but may provide more predictable results?**

- **Participant #7:** Why "versus"? Why not have a diverse portfolio?

- **Participant #12:** I am leaning towards the innovative, more risky research. Isn't that the point?

- **Participant #15:** There will be research not funded by the NIH - like developing screens for drugs. Maybe it is boring, but it will develop tools to advance the field.

- **Participant #12:** To me, we need to lean in the direction of the innovative.

- **Participant #5:** Whether it is risky or not, whether it's done by NIH or CIRM, the fundamentals of the research activity may be the same. Just because the goals are innovative or risky doesn't mean they don't go through the same research process.

- **Participant #12:** It seems there are a lot of arenas where things can happen in four months rather than eight months, and that is the niche for this group. There are a lot of instances where the timeline doesn't have to be eight months.

- **Participant #4:** There are also some areas where they have a hard time getting funding. For example, we have to use private funding to care for rats because we cannot get government funding to keep a chronically injured rat alive; research isn't just focused on the acute but things like that don't fall into either category.

- **Participant #2:** Why would we close the door on any kind of research? Minimally risky research may be part of one avenue of research. Plus, there are many definitions of risks - there is risk to the patient, there is political risk. I say don't close the door to any element of research that may move things forward.

- **Zach Hall:** Let's say you are evaluating grants and you have two in front of you, but you only have money to fund one. It's like the stock market - do you put your money into emerging markets or bonds? What are your concerns about? Do you care more about results or stability? You don't know if this project will work or not; if it works it will really make a difference, but you will need to take a chance. The problem is if you bet on a risky project and it fails, when you approach your milestones you have to say it did not work. These decisions are like the stock market in that the big payoffs often come with great risk.

- **Participant #7:** Why can't it be that we use 70% of our funding on risky research and the rest for more predictable, minimal risk research?

- **Zach Hall:** At the time of the Human Genome Project, there were two proposed approaches to sequencing the genome. Someone who advocated the approach that ultimately wasn't

chosen left the NIH to set up a competing project. That was Craig Ventner. The competitive pressure he exerted caused the NIH to change its strategy and accomplish things much faster. Some say they wouldn't have done that without competition from Craig Ventner. It's true the NIH study sections put a big priority on feasibility and likelihood of return. I don't know that we look to you for an answer but we do listen for whether it's more important to have tangible progress or are we willing to risk some of the money on a gamble that may fail.

- **Participant #8:** We should embrace a flexibility that will allow us to capitalize on unanticipated results.

- **Zach Hall:** Maybe we should substitute "opportunistic" for your earlier term "scientific readiness"?

- **Participant #10:** Also throw in "leverage".

- **Participant #18:** Embedded in this discussion is the idea that there are parts of the NIH process that are cumbersome and inflexible. One of our goals is a dynamic grants process. If researchers propose an experiment but come back with results that lead in a different but productive fashion, at the NIH, they would be stalled. We can incorporate a dynamic process that accepts an organic movement of this process so that it's always moving. The milestones can be reset in a way that moves in the direction of the grant. We have to ask: how will we improve the speed of scientific advancement and the ability to capitalize on changes in direction so we don't cripple science as it dynamically and spontaneously moves forward?

- **Participant #17:** A number of us in the patient community are looking for you to be more risky and innovative. Let us make the moral decision about doing this with our children and putting them into clinical trials.

- **Participant #9:** I feel a little uncomfortable with investing 70% of the funds in risky projects. We need some kind of balance and I'm not sure if we are looking at [the right] percentages. Science and medicine has been built on the methodical.

- **Participant #6:** We don't necessarily want it to be 70/30. It could be 50/50, or it could flexible and dynamic. We are looking at attitudes and risk tolerance. I have seen the research at established institutions and I wish more would fall into the risky and innovative category. Proposition 71 could serve as a prod to get a dynamic, intelligent group of people to start thinking about more risky and innovative research.

Patient Involvement / Participation

7. **How should the CIRM partner with disease specific organizations/non-profits?**

8. **What is the best way to educate the public about the progress of the research? (Eg. Annual meetings, web site, an independent group (like AFSCR), periodic reports?)**

- **Participant #11:** The Leukemia and Lymphoma Society has been able to fund different researchers from different hospitals and areas to bring about medications for at least leukemia. Five years ago, the only drug out there for leukemia was interferon. Now there are two drugs (Gleevec and Spricell) that are molecularly based that have been researched and gone through the FDA fast track, so things can be done in a short period of time.

- **Patricia Olson:** How did you find out about those?

- **Participant #11:** The way that I found out about these drugs was to start with the internet, do research, and go to a website that looks at clinical trials and look at how I could be more connected.

- **Patricia Olson:** How could CIRM facilitate that process?

- **Participant #11:** If CIRM got together with specific organizations like the Leukemia and Lymphoma Society and put together packets that could be taken to and distributed at hospitals and cancer centers, the public can find out what is going on. It is extremely important that the general public be educated on what is going on.

- **Participant #13:** Our websites give you a vehicle where if progress is updated and transmitted routinely, we can plug it into our websites and newsletters. This has to be something created by CIRM because this will not work for us if we have to summarize and condense it down. Give us two lean, concise paragraphs that can be plugged in. You could reach millions of people.

- **Participant #8:** Those groups are a source of patients for clinical trials; any one of these associations is a major source for that. Most associations have a VPs of Science and that group of people should be identified in some database that CIRM keeps so you can check out all kinds of things that come through CIRM on a particular disease. Most likely that VP of Science will know the background of that and where it is going.

- **Patricia Olson:** So it's a two way process - we work with your organization to understand your perspective and we provide updated information specific to your organization?

- **Participant #5:** For the first question [about partnering with disease specific organization] I would say a quarterly update meeting like this focus group. This can provide an update on projects, allow interchanging of ideas, and communication of successful actions. For the second question [about educating the public on your progress], the best way is through websites, an annual meeting, and definitely the use of newsletters that could go out broadly to the memberships of any of the nonprofits. This newsletter could be a little more generic and focused on the lay person to give an overview of what CIRM is doing, what some of the non profits are doing, and any breakthroughs. This would alleviate the need of a broad, extensive report of 50-60 pages. A newsletter could be 6-8 pages and easy to read through.

- **Participant #12:** Maybe if we can get your press releases too.

- **Participant #3:** We do a perfectly good job of collaborating with disease organizations informally. Maybe we can have a collaboration modeled after CAMR [Coalition for the Advancement of Medical Research] or do some type of sub-linkage through CAMR. Remember, educating the whole public is not our responsibility. Remember the value of expediency. We are not going to get everyone and we want to get politics out of this.

- Participant #9: We still owe something to the public. To me, the greatest hero is Lance Armstrong because of what he has been doing with clinical trials in cancer. If we could find a spokesperson that that public at large can relate to, that would be huge. The public wants to know that we are good stewards of this bond.

- **Participant #7:** If we take all the organizations in this room, that is the public, and if we cover all the people on all those mailing lists, we've informed the public.

- **Participant #2:** I like the idea of sharing with disease specific organizations. Maybe we can have an annual conference in collaboration with Stanford or UCSF that is open to the public, researchers, and healthcare professionals. We would inform them who is doing what in what area. This kind of thing can bring people in.

- **Participant #12:** We can also do that through Webcasts and Webinars.

- **Participant #13:** We also have to remember the diversity of our state. We need to think about how we can spread this news to all populations. Some issues are specific to certain ethnicities. Certain cities also have language issues. I don't want these issues to get lost.

- **Zach Hall:** We are devoting a focus group to discuss this.

9. **How can the CIRM educate and inform patients (the public) so they can have realistic expectations?**

10. **How can the public/patient community be prepared for and educated about the risks/benefits of clinical trials?**

- **Patricia Olson:** We need to keep in mind that the level of regulation is not decreasing. It may not be increasing, but it's there.

- **Participant #5:** Both these questions can easily be dealt with through the media, such as public service announcements or a newsletter. Regarding clinical trials, that could be done through a written pamphlet distributed to the medical community. It would give patients another perspective and could be a way to educate the community. It is also a way to communicate what the state is doing for the residents and citizens of California to alleviate disease. It would be a good public message. The repetitiveness of it would be sustained and it would generate good will.

- **Participant #17:** I agree with using the press, but sometimes some of the information has not been picked up. We may need to develop relationships with the reporters. We also need to be careful not to release anything too prematurely.

- **Participant #4:** The patient community is pretty much aware of the risks and benefits of clinical trials. Some patients are desperate. What we have to do is let the scientists know that we are willing to take the risks and let the public know that failure is OK. In the spinal cord community, 10% of spinal cord patients are committing suicide every year. We have to accept failure and scientists cannot be afraid of it.

- **Patricia Olson:** I am not sure if it is an issue of failure in clinical trials, but of safety. We cannot forget the regulations and our responsibility to the safety of patients.

- **Participant #4:** People have to be educated to the fact that patients are willing to take risks.

- **Participant #10:** We need to let the doctors know this too. They are in the forefront.

- **Participant #12:** Some patients don't trust the healthcare system and would not want to participate in clinical trials. As for the first question, with regard to pharma, because my organization doesn't want to hear information about new advances in the media first and then have to deal with questions we are not prepared to answer, a lot of times, the pharmaceutical companies will hold a conference call with us before things go to the media so that we have a chance to respond to constituents. It gives us a little advance warning.

- **Participant #18:** If we can focus on how the public can be prepared, that would be valuable. The patient advocacy community is in many cases highly informed, but for the intellectual and political leadership of the public as well as the legislative and business leadership, if there are failures in the early clinical trials, and statistically there will be, we have to anticipate that there will be groups that will immediately exploit this. We can take on the responsibility with the patient groups of informing the legislative and business leadership of the risks and the acceptance of the risks. If they begin to understand that risks are responsibly assumed and are expected as part of this process to advance medicine and mitigate suffering, if and when predictive failures exist, this won't fall back on us. We have to accept joint responsibility for preparing the political and business leaders.

- **Participant #5:** The issue is educating the medical community so that they are on board whole heartedly, which we hear they are not. These pamphlets we discussed should communicate to the patients and medical community the scope of this endeavor so that they understand that this endeavor assumes the same risks as many other types of ventures. It is a venture that the state is taking but it's a calculated risk, as anything is. To reduce health costs, we need to get people on board. We need to solicit and recruit patients on clinical trials. We could maybe partner with California Health Association to do this. This message must be communicated.

- **Participant #2:** In my experience, physicians are probably the most difficult to educate. If we are proactive in getting the word out early, quickly, and in a meaningful way so that we can talk about it without being put on the spot, that would be huge.

- **Participant #10:** Knowing that there will be failures or learning curves, we need to educate the public and get those message points out before anything going out to the clinic.

11. As a patient, if you could say one thing to the CIRM about the strategic plan, what would it be?

- **Participant #5:** If there is one message that should be gotten across to the state of California and to the general public, it's that we need to put a human face on this issue. That would be the primary thing for the strategic plan - that the face of this issue is a human face.

- **Participant #3:** Go forth and do it. Do what no one else can do. Bridge the gaps.

- **Participant #4:** Time is of the essence.

- **Participant #10:** I always highlight the children, but this issue cuts across the spectrum. Also, never say no, just ask how.

- **Participant #17:** Move faster, move forward, and look at what we can come up with quickly.

- **Participant #18:** Of the unique assets we need to develop, the internet communications capacity of the patient advocacy groups is an extraordinary high quality way to pass information to the society. We have underutilized it and do not have existing, in-depth linkages to it. We need to be able to transmit quickly, in real time, high quality messages through these systems. This would provide a tremendous communication tool at low cost.

- **Participant #7:** Don't become another government bureaucracy. We don't care about the government process. Innovate. Keep letting us know why we should provide support.

- **Participant #9:** People want cures immediately and we know that cannot happen. We have to show accountability. It is important that we look at internal measures and metrics, or we will be seen as a failure if cures aren't there.

- **Participant #8:** Hurry up and maintain a sense of urgency that the rest of us feel.

- **Participant #2** I want to remind everyone that 20 years ago IVF was controversial and now it is not. As we move forward as long as our integrity stays high, we will move forward.

- **Participant #13:** Utilize all of us.

- **Participant #1:** We need to look to be ambitious and aggressive, and go.

- **Participant #15:** It is important to focus on broad based ways to support the field and give researchers the tools to run with their curiosity and talents to move forward.

- **Participant #6:** Time is of the essence.